NIH Collaboratory

Health Care Systems Research Collaboratory

Introduction to pragmatic clinical trials

How pragmatic clinical trials bridge the gap between research and care

Overview

THE BIG PICTURE

- Major challenges related to traditional clinical research—and the National Institutes of Health's vision for bridging the gap between research and care

A MORE PRACTICAL APPROACH

- Pragmatic clinical trials—what are they and what are their advantages over traditional randomized trials in terms of relevance and applicability to everyday practice

THE PAYOFF

- How health care systems, providers, and patients will benefit by partnering in pragmatic clinical research

Challenge #1: Clinical research is slow

- To most people, randomized controlled trials (RCTs) are the mainstay of clinical research.
- But traditional RCTs are slow and expensive—and rarely produce findings that are easily put into practice.

- In fact, after an **average of 17 years** only 14% of research findings will have lead to widespread changes in care.[1]

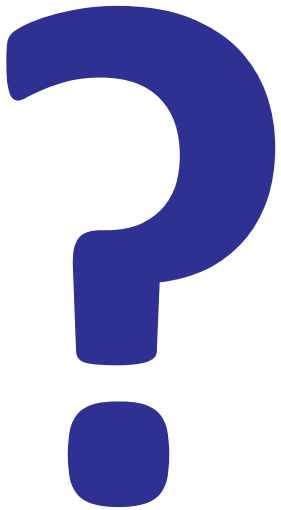
Challenge #2: Clinical research is not relevant to practice

- Traditional RCTs study the effectiveness of treatments delivered to carefully selected populations under ideal conditions.
- This makes it **difficult to translate results to the real world**.
- Even when we do implement a tested intervention into everyday clinical practice, we often see a "voltage drop"—a dramatic decrease in effectiveness.

> "If we want more evidence-based practice, we need more practice-based evidence."
>
> Green, LW. *American Journal of Public Health*, 2006.

Challenge #3: The evidence paradox

- More than 18,000 RCTs are published each year—in addition to tens of thousands of other clinical studies.
- Yet systematic reviews consistently find that **we don't have enough evidence to effectively inform the clinical decisions** providers and patients must make.



We need a more practical, more integrated approach

- Clinical research is more than just traditional RCTs.
- **Pragmatic research** is designed with input from health systems—and produces evidence that can be readily used to improve care.
- By engaging health systems, providers, and patients as partners, pragmatic research accelerates the integration of research, policy, and practice.

The vision: Partnerships that support faster, more relevant research

"**Partnerships with health care systems** offer an opportunity to transform research and ultimately improve America's health. "

"Working together, we can achieve our common goal: **speeding the movement of scientific discoveries from the lab to patients**."

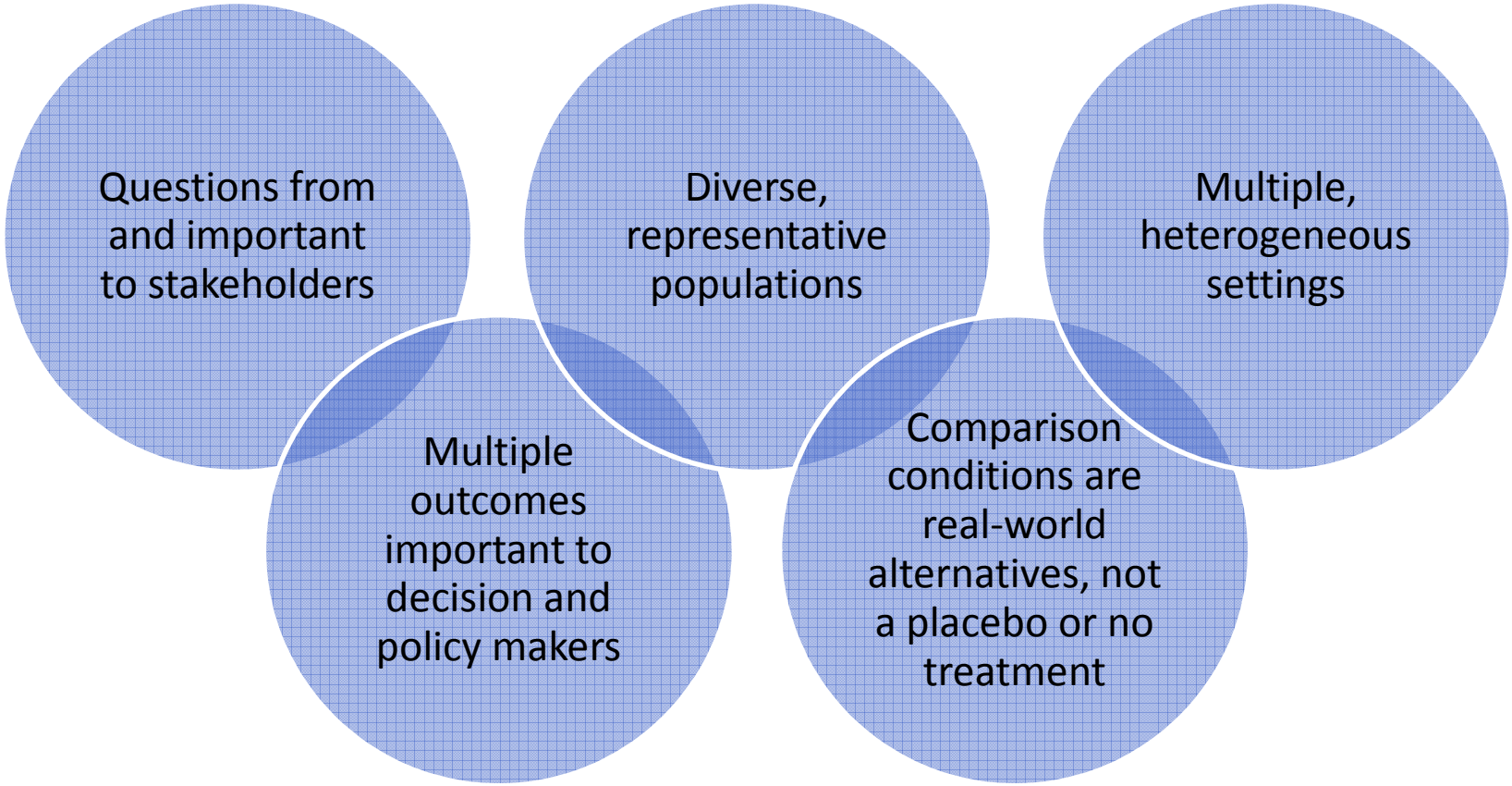
– NIH Director Francis Collins, MD, PhD

Pragmatic clinical trials: Partnership-based research built to improve care

- Pragmatic clinical trials (PCTs) are designed to **improve practice & policy**.
- Unlike most traditional RCTs, they take place in settings **where everyday care happens**, such as community clinics, hospitals, and health systems.
- **Collaborating providers and organizations are integral partners** and gain practical evidence on how to improve patient health and satisfaction.

- **Pragmatic partnerships engage at multiple levels** – including patients, practitioners, teams, health systems and communities.

Core characteristics of pragmatic clinical trials (PCTs)[2-4]



Questions health care teams might ask before participating in any clinical trial

	Traditional RCTs	Pragmatic Clinical Trials
Is it important to us?	?	You and your colleagues help formulate the research question(s).
Can we do it here?	?	The study is built around your normal health care operations.
Will it take us more time?	?	Flexible study protocols minimize intrusion in your daily work flow.
Will it help our patients?	?	The study's explicit goal is to give you evidence that improves patient care & clinical decision making.

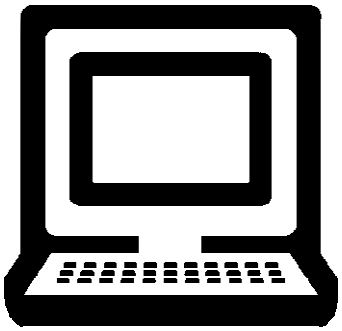
Key differences between RCTs &PCTs

	A traditional RCT tests a hypothesis under ideal conditions	A PCT compares treatments under everyday clinical conditions
GOALS	To determine causes and effects of treatment	To improve practice and inform clinical & policy decisions
DESIGN	Tests the intervention against placebo using rigid study protocols & minimal variation	Tests two or more real-world treatments using flexible protocols & local customization
PARTICIPANTS	Highly defined & carefully selected	More representative because eligibility criteria are less strict
MEASURES	Require data collection outside routine clinical care	Brief and designed so data can be easily collected in clinical settings
RESULTS	Rarely relevant to everyday practice	Useful in everyday practice, especially clinical decision making

What are the benefits? PCTs are…

Practical	• Designed to test what will work in everyday care, with emphasis on successful implementation.
Inclusive	• PCTs study diverse populations receiving care in real-world settings using broadly inclusive criteria for study participation.
Engaged	• Health systems, providers, and patients are involved in study design, collecting data, interpreting results, and acting on findings.
Relevant	• Results designed to directly inform decision-making of administrators, providers, patients, and policymakers.

Common pragmatic research features



Use of electronic health records (EHRs)

- EHRs allow efficient and cost-effective, recruitment, data collection, and participant communication, monitoring, & follow up.

Randomization of treatment alternatives based on normal health care operations

- This sometimes mean randomizing at the clinic or provider level (“cluster randomization”).

PCTs: Fewer exclusions allow for a broader subset of participants

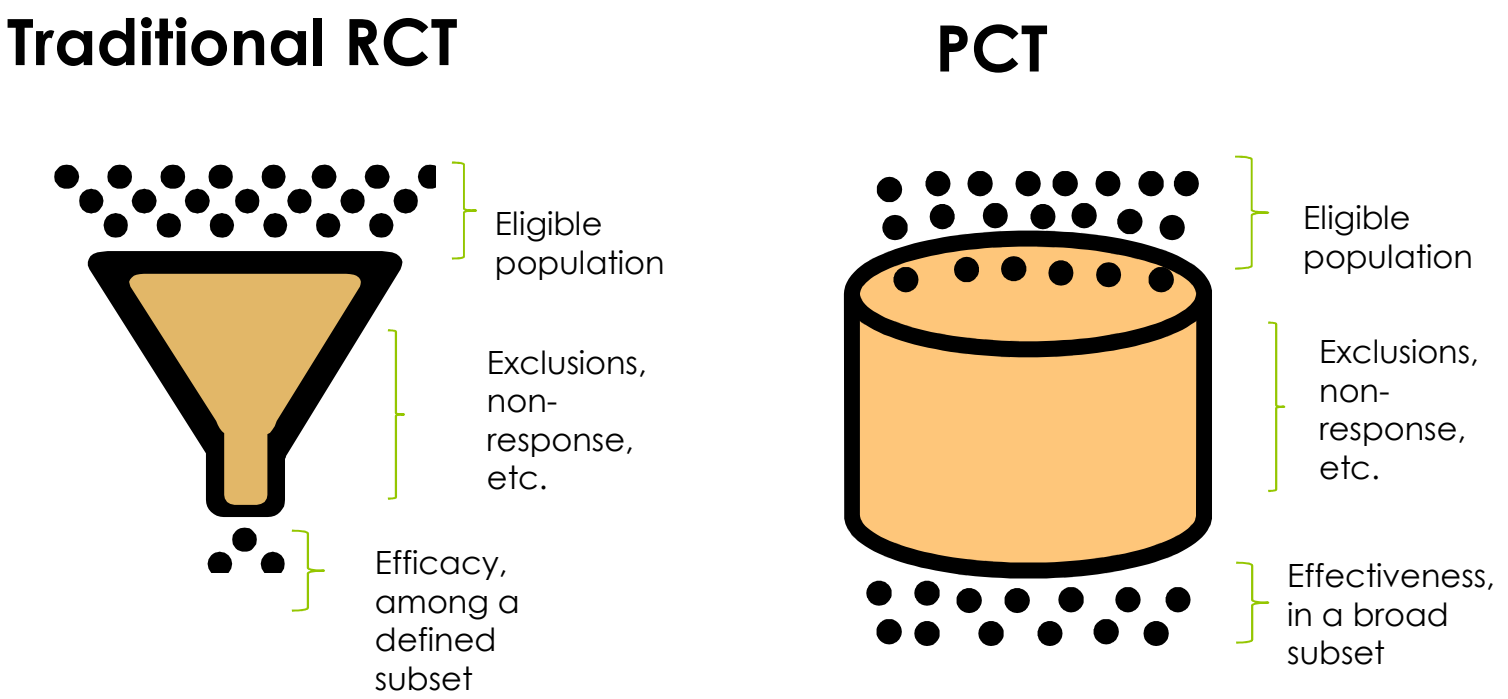
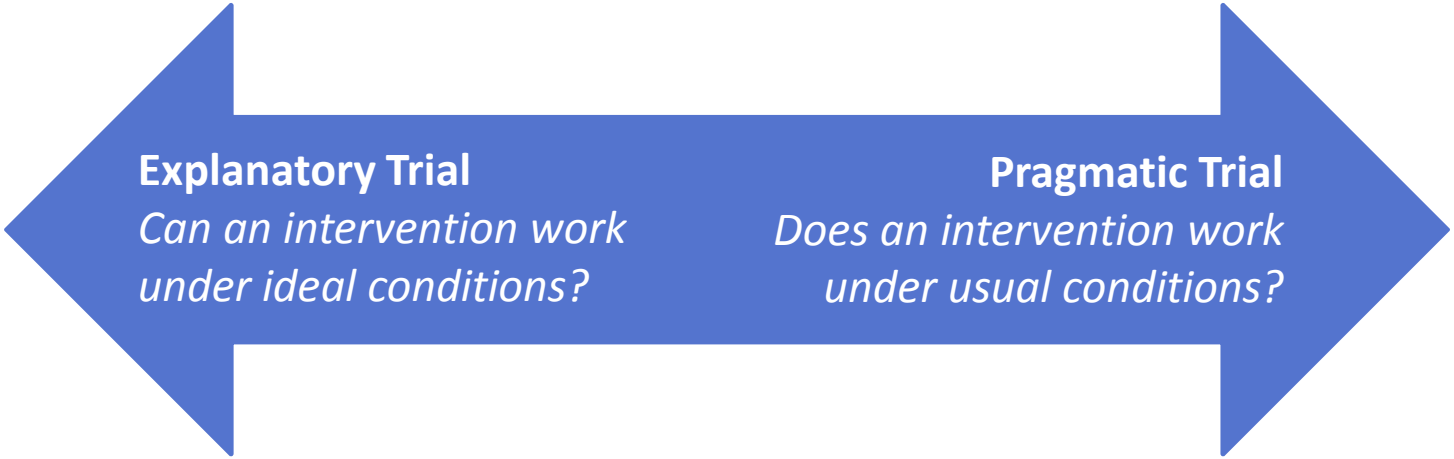


Figure provided by Gloria Coronado, PhD, Kaiser Permanente Center for Health Research

The RCT-PCT continuum

- PCTs are not an abandonment of the scientific methods that have lead to countless breakthroughs.
- They don't take away from basic science or diminish the importance of traditional RCTs—we just need a balance.
- **No clinical trial is completely explanatory or pragmatic.** RCTs and PCTs exist on a continuum.



Pragmatic trials have been benefitting health care for decades

1950s

- Polio vaccine studies

1980s & 90s

- Early studies of acute treatment for heart attack

Growing recognition that more pragmatic research is needed

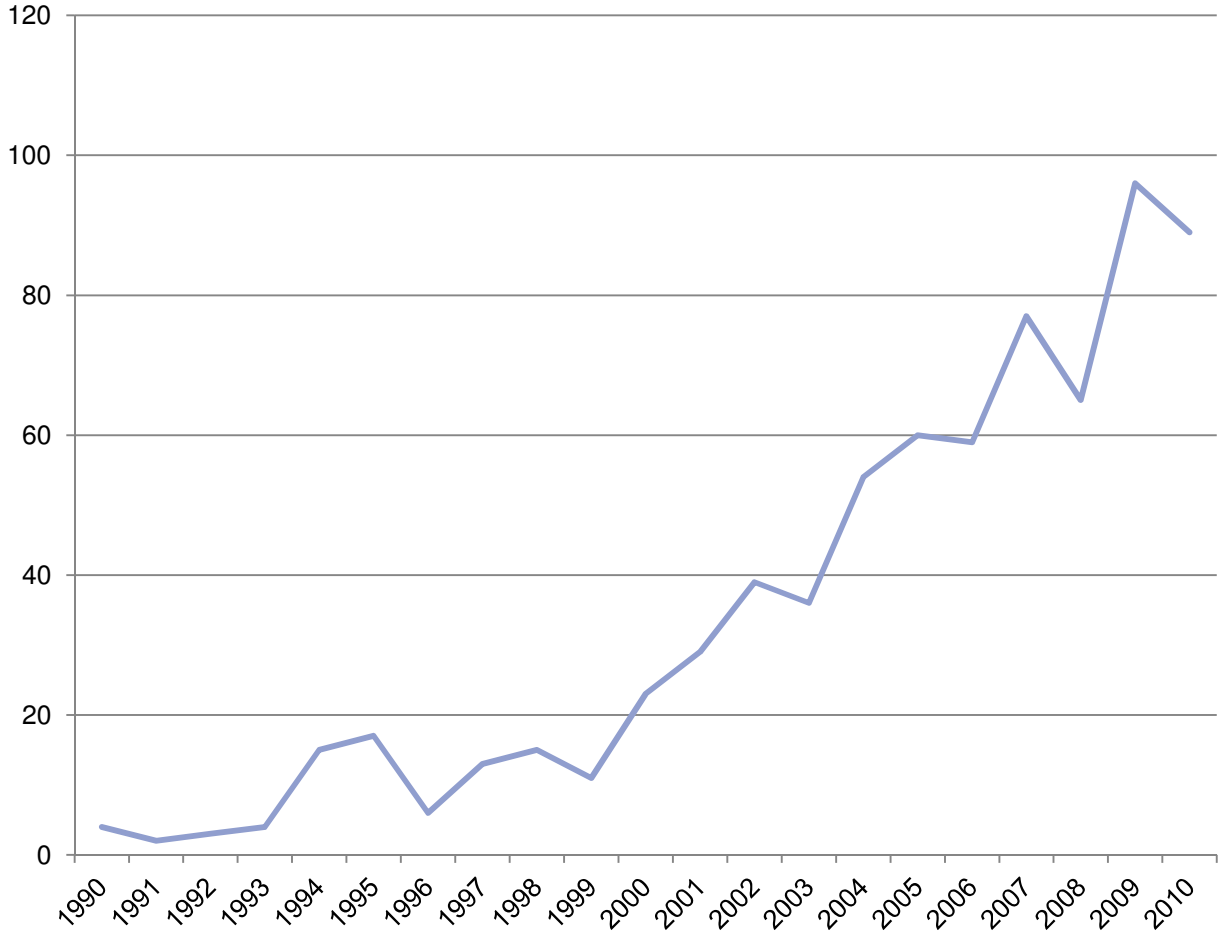


Figure provided by Sean Tunis, MD, Center for Medical Technology Policy

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Pragmatic trials promote learning health care systems

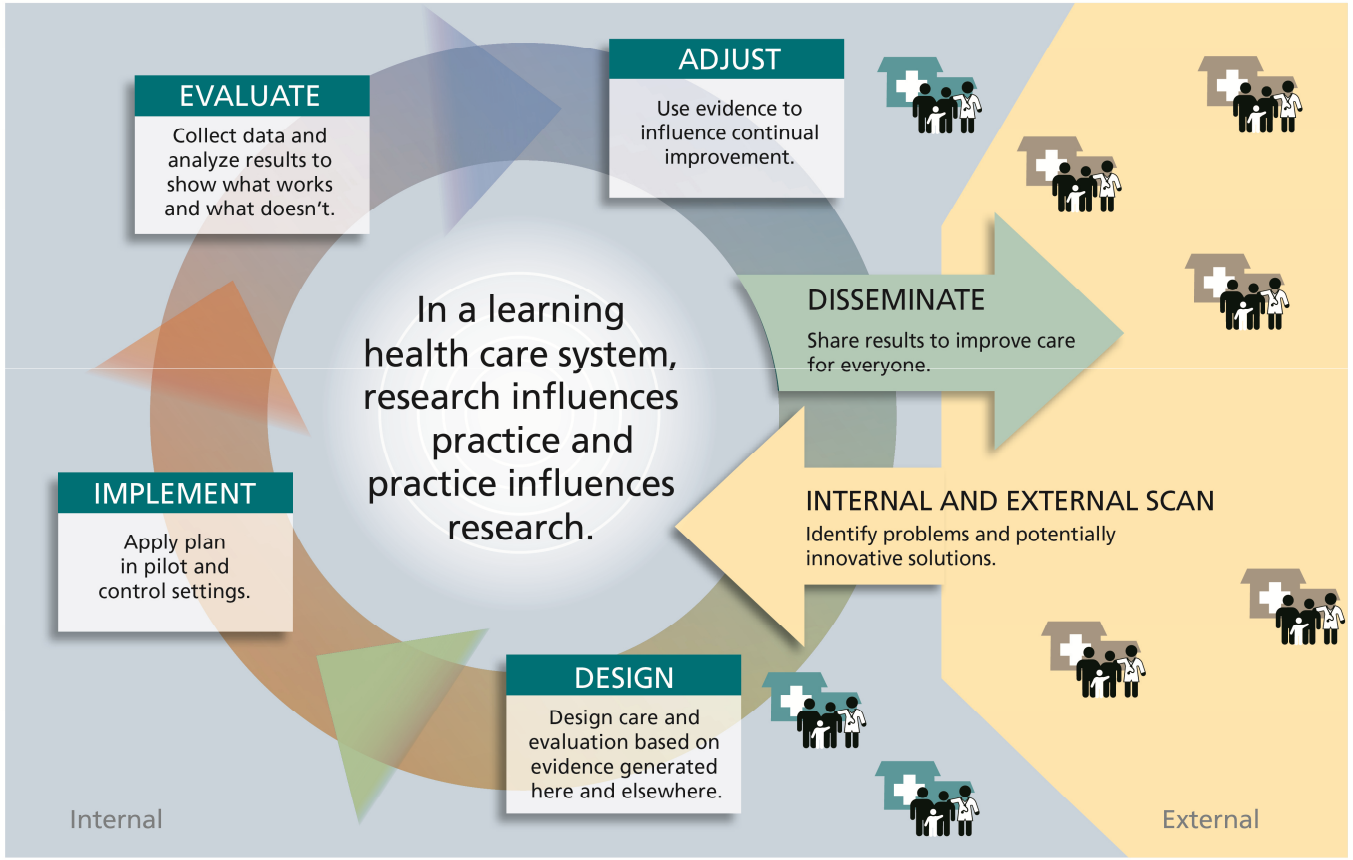
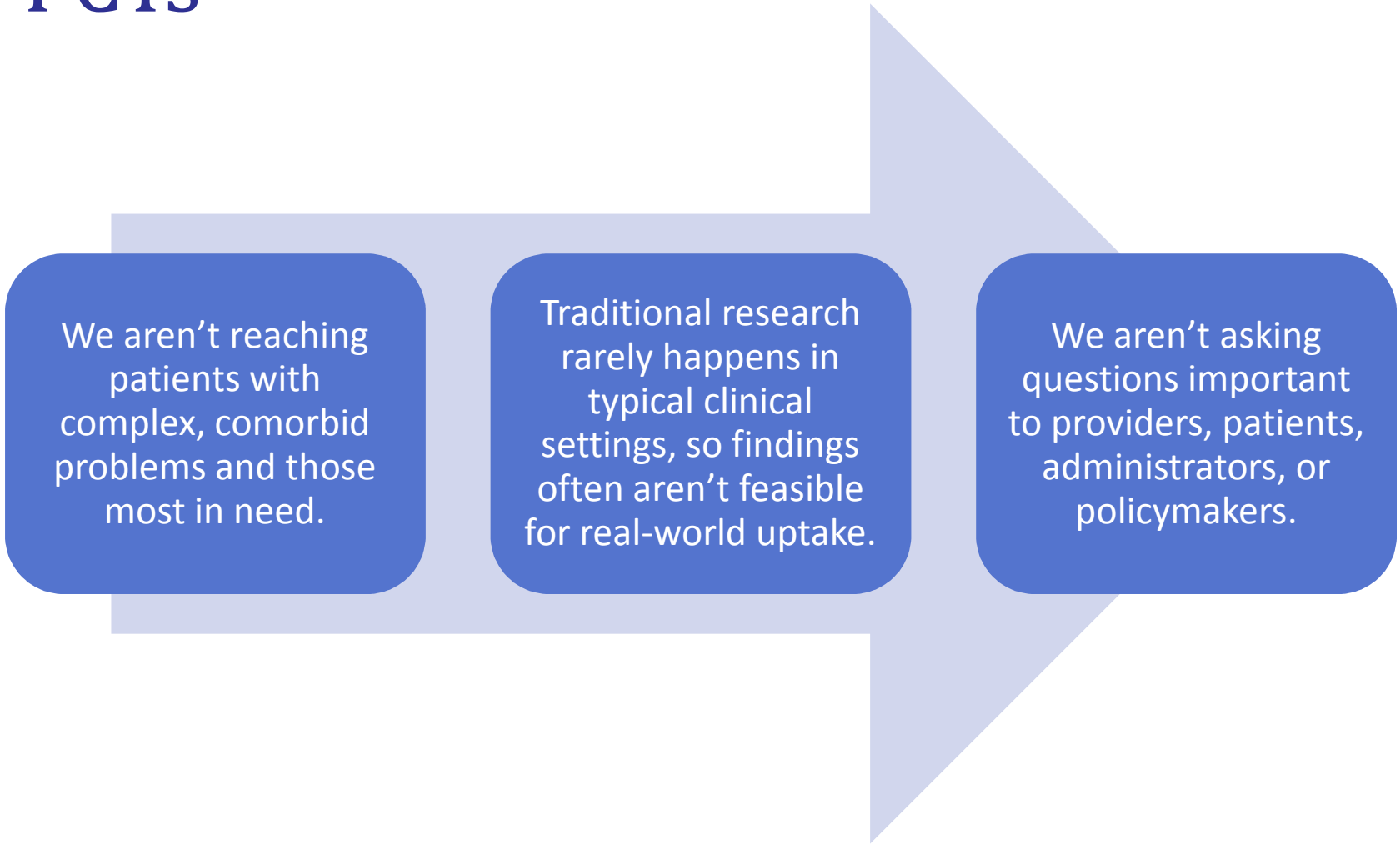
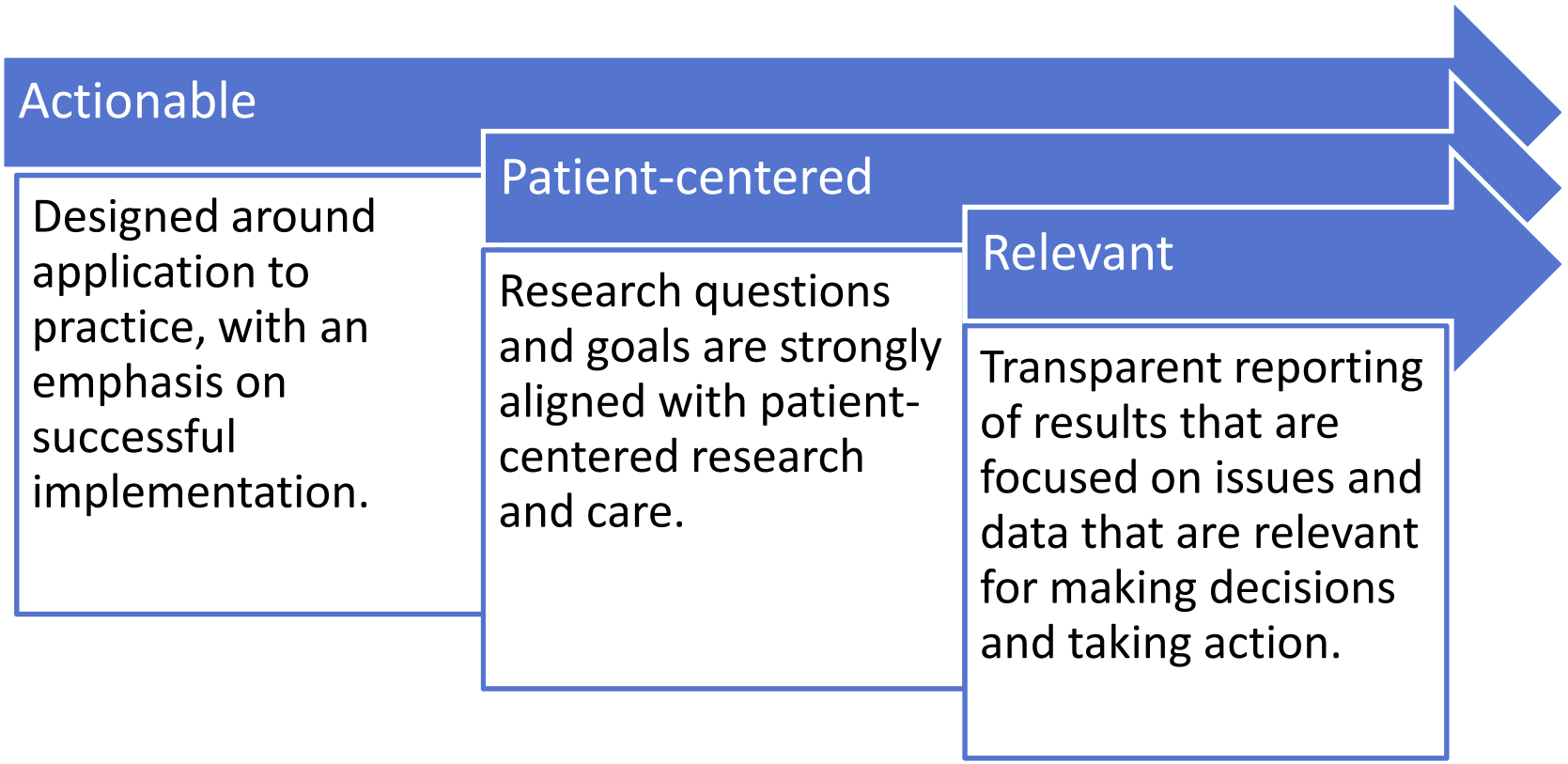


Figure from Greene SM, et al. Annals of Internal Medicine, 2012. Used with permission.

Take-home messages: Why we need PCTs



Take-home messages: Benefits of PCTs for healthy systems & providers



Questions for discussion

- Tip–when presenting on pragmatic clinical trials to health systems, be sure to elicit their perspective. Getting input from a range of health care personnel as well as researchers is critical to designing and implementing pragmatic clinical trials.
  - **What is on your mind**? What are clinical or operational issues that have been struggles and where new answers would help?
  - **What is going on?** What is happening in environment (for example a new payment approach or treatment) that might serve as a natural experiment?

Key references and resources

1. Balas EA, Boren SA. Managing clinical knowledge for health care improvement. In: Bemmel J, McCray AT, editors. Yearbook of Medical Informatics 2000: Patient-Centered Systems. Stuttgart, Germany: Schattauer Verlagsgesellschaft mbH; 2000:65-70.
2. Thorpe KE, et al. A pragmatic-explanatory continuum indicator summary (PRECIS): a tool to help trial designers. *Can Med Assoc J*, 2009, 180: E47-57.
3. Tunis SR, et al. Practical clinical trials: increasing the value of clinical research for decision making in clinical and health policy. *JAMA,* 2003;290:1624-1632.
4. Glasgow RE, et al. Practical clinical trials for translating research to practice: design and measurement recommendations. *Med Care,* 2005;43(6):551-557.
5. Zwarenstein M, et al. Improving the reporting of pragmatic trials: an extension of the CONSORT statement. *BMJ*, 2008 Nov 11;337:a2390.

Prepared by The NIH Health Care Systems Research Collaboratory

- $11.3 million from NIH to engage health care organizations as research partners in large-scale studies designed to yield results relevant to clinical practice.
- Seven demonstration projects were funded in 2012—and all include partners from health care systems, provider organizations, and community health centers.
- More projects to come…

> "The Health Care Systems Research Collaboratory…will move us beyond traditional randomized clinical trials to more broad-based, real-world settings."
>
> - NIH Director Francis Collins, MD, PhD

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